



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2015-N-0430]

Measuring Dystrophin in Dystrophinopathy Patients and Interpreting the Data; Public Scientific Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public scientific workshop; request for comments.

The Food and Drug Administration (FDA) is announcing a public scientific workshop to discuss dystrophin protein quantification methodologies for human tissue. This workshop is being cosponsored by the National Institutes of Health (NIH). The purpose of the workshop is to discuss currently available methodologies and to identify scientific knowledge gaps and opportunities for improving dystrophin protein detection in the context of drug development. The intended audiences for this workshop are scientists and clinicians involved in the acquisition, measurement, and analysis of proteins associated with Duchenne Muscular Dystrophy (DMD).

Dates and Time: The scientific workshop will be held on March 20, 2015, from 8:30 a.m. to 5:30 p.m.

Address: The scientific workshop will be held at FDA's White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (rm. 1503A), Silver Spring, MD, 20993-0002. Participants must enter through Building 1 and undergo security screening. For parking and security information, please refer to

<http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm>.

Contact Persons: Mary Gross, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-796-3519, [mary.gross@fda.hhs.gov](mailto:mary.gross@fda.hhs.gov); or Georgiann Ienzi, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-796-3515, [georgiann.ienzi@fda.hhs.gov](mailto:georgiann.ienzi@fda.hhs.gov).

If you need special accommodations due to a disability, contact Mary Gross or Georgiann Ienzi at least 7 days in advance.

Registration: The scientific workshop is free and seating will be on a first-come, first-served basis. It may be necessary to limit both the number of attendees from individual organizations and the total number of attendees based on space limitations. Email registrations should be sent to [Dystrophin\\_Workshop@fda.hhs.gov](mailto:Dystrophin_Workshop@fda.hhs.gov) by March 17, 2015. If you cannot attend in person, the meeting will be Webcast live. Information about how to access the Webcast will be located at: <http://www.fda.gov/Drugs/NewsEvents/ucm432429.htm>.

Comments and Meeting Summary: Submit electronic comments to <http://www.regulations.gov> by May 20, 2015. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. Please identify your comments with the docket number found in brackets in the heading of this document. It is only necessary to send one set of comments. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at <http://www.regulations.gov>.

A summary of the scientific workshop's highlights will be made available for review at the Division of Dockets Management and at <http://www.regulations.gov>. You may submit a request to obtain a hard copy by sending a request to the Division of Freedom of Information

(ELEM-1029), Office of Management Programs, Food and Drug Administration, 12420 Parklawn Dr., Element Bldg., Rockville, MD 20857.

SUPPLEMENTARY INFORMATION: FDA and NIH are cosponsoring this scientific workshop to discuss current methodologies being used in drug development and scientific research for DMD. Recent scientific advances present an opportunity for the development and validation of robust methods for the objective, reliable, and quantitative measurement of DMD-associated proteins.

## I. Background

Dystrophinopathies result from genetic mutations in the dystrophin gene that decrease dystrophin protein expression levels and result in altered dystrophin function. These changes can lead to muscle degeneration and, in many patients, downstream pathologies including inflammation and fibrosis that interfere with muscle regeneration, loss of movement, orthopedic complications, and ultimately respiratory and cardiac failure.

## II. Scope of the Scientific Workshop

The workshop will include sessions which will focus on current technologies used in the detection of dystrophin. Presentations will provide overviews of the technologies (including limitations, detection sensitivities, linearity, and reproducibility). A panel discussion will help identify development challenges for each method. Muscle biopsy collection, sample handling, reference materials, and image analysis will also be discussed.

FDA will post the agenda and other background material approximately 2 days before the public scientific workshop at: <http://www.fda.gov/Drugs/NewsEvents/ucm432429.htm>.

Dated: February 24, 2015.

Leslie Kux,

Associate Commissioner for Policy.

4164-01-P

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